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Dear Duchenne Families,

Today we shared our plans for the launch of EMFLAZA™ (deflazacort), the first FDA-approved corticosteroid for Duchenne muscular dystrophy patients age 5 and older, regardless of the genetic mutation. We are excited to launch EMFLAZA in the U.S., and we will begin making the product commercially available to Duchenne patients and families in the upcoming weeks. We have a team in place with extensive experience, including a dedicated field force to support Healthcare professionals and the patient community.

For nearly 20 years, PTC has been working to understand how to investigate Duchenne muscular dystrophy and to identify therapies that fundamentally change the lives of patients living with this disorder. The launch of EMFLAZA is another step toward achieving this goal. The approval of EMFLAZA for all Duchenne patients regardless of genetic mutation provides us with the opportunity to work with the whole Duchenne community to help standards of care. We will do this through awareness initiatives to enable diagnosis and access to treatment and specialty care.

We know some families are anxious about the affordability and access to EMFLAZA. In response, we have set a goal to provide EMFLAZA to all eligible Duchenne patients, regardless of insurance status or type, and have developed a process so that patients have seamless access to the appropriate service. We have a trained team of case managers at EMFLAZA*Cares*™ who will work with Duchenne patients every step of the way to help them get EMFLAZA with minimal difficulty and expense.

We appreciate that prior to FDA approval, access to deflazacort had been difficult and many patients were unable to get the drug. Our team at PTC is ready to deliver a consistent and reliable supply of EMFLAZA and has high quality assurance measures in place to oversee and manage production and inventory.

We are proud of our almost two decade involvement with the Duchenne community, and we are looking forward to taking this next step in our journey with you. From all of us at PTC, we thank you for your partnership in helping us bring this important therapy to Duchenne patients in the U.S.

Please see Indication and Important Safety Information below.

Please see link to full Prescribing Information www.EMFLAZA.com.

Sincerely,

Stuart Peltz
Chief Executive Officer

INDICATION & IMPORTANT SAFETY INFORMATION FOR EMFLAZA™ (deflazacort)

INDICATION
EMFLAZA is a corticosteroid indicated for the treatment of patients with Duchenne muscular dystrophy (DMD) in patients 5 years of age or older.

IMPORTANT SAFETY INFORMATION

Contraindications: EMFLAZA is contraindicated in patients with a hypersensitivity to deflazacort or any of the inactive ingredients in EMFLAZA

Warnings & Precautions

• **Alterations in Endocrine Function:** Corticosteroids, such as EMFLAZA, can cause serious and life-threatening alterations in endocrine function, especially with chronic use. Monitor patients receiving EMFLAZA for Cushing's syndrome, hyperglycemia, and adrenal insufficiency after EMFLAZA withdrawal. In addition, patients with hypopituitarism, primary adrenal insufficiency or congenital adrenal hyperplasia, altered thyroid function, or pheochromocytoma may be at increased risk for adverse endocrine events. Acute adrenal insufficiency or "withdrawal syndrome" can occur if corticosteroids are withdrawn abruptly, and can be fatal. The risk is reduced by gradually tapering the corticosteroid dose when withdrawing treatment. During times of medical stress, corticosteroid dosage may need to be increased.

• **Immunosuppression and Increased Risk of Infection:** Increased risk of new, exacerbation, dissemination, or reactivation of latent infections, which can be severe and at times fatal; Signs and symptoms of infection may be masked. Tell patients and/or caregivers to inform their healthcare provider if the patient has had recent or ongoing infections or if they have recently received a vaccine. Warn patients who are on corticosteroids who have not had chickenpox or measles to avoid exposure to chickenpox or measles and to alert their healthcare provider immediately if they are exposed.

• **Alterations in Cardiovascular/Renal Function:** Monitor for elevated blood pressure. Dietary salt restriction and potassium supplementation may be needed.

• **Gastrointestinal Perforation:** Increased risk of gastrointestinal perforation during corticosteroid use in patients with certain gastrointestinal disorders such as active or latent peptic ulcers, diverticulitis, recent intestinal anastomoses, and inflammatory bowel disease. Signs and symptoms may be masked.

• **Behavioral and Mood Disturbances:** May include euphoria, insomnia, mood swings, personality changes, severe depression, and psychosis. Encourage patients to seek medical attention if symptoms develop or worsen.

• **Effects on Bones:** The risk of osteoporosis increases with prolonged use of EMFLAZA, which can predispose patients to vertebral and long bone fractures. Monitor for decreases in bone density with chronic use of EMFLAZA.

INDICATIONS & IMPORTANT SAFETY INFORMATION FOR EMFLAZA (deflazacort)

• **Ophthalmic Effects:** May include cataract formation, ocular infections, and glaucoma. If treatment with corticosteroids, including EMFLAZA, are continued for more than 6 weeks, monitor intraocular pressure.

• **Vaccination:** Do not administer live or live attenuated vaccines to patients receiving immunosuppressive doses of corticosteroids. Killed or inactivated vaccines may be administered, but the responses cannot be predicted.

• **Serious Skin Rashes:** Toxic epidermal necrolysis has been reported with the use of deflazacort. Discontinue at the first sign of rash, unless the rash is clearly not drug related.

• **Effects on Growth and Development:** Long-term use of corticosteroids, including EMFLAZA, may slow growth and development in children.

• **Thromboembolic Events:** Observational studies have shown an increased risk of thromboembolism. Use EMFLAZA with caution in patients who have or may be predisposed to thromboembolic disorders.

Adverse Reactions: The most common adverse reactions (2:: 10% for EMFLAZA and greater than placebo) are Cushingoid appearance, weight increased, increased appetite, upper respiratory tract infection, cough, pollakiuria, hirsutism, central obesity, and nasopharyngitis.

Drug Interactions: Give one third of the recommended dose of EMFLAZA when EMFLAZA is administered with strong or moderate CYP3A4 inhibitors. Avoid use of strong or moderate CYP3A4 inducers with EMFLAZA, as they may reduce efficacy.

Please see www.emflaza.com for the full Prescribing Information.

To report adverse events, please contact ProPharma Group at 1-866-562-4620 or drugsafety@propharmagroup.com.

You may also report adverse events to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.